A Hopeful Future for Rare Disease in Canada

Presented by Takeda Better Health, Brighter Future

On March 3rd, 2022, Canada 2020, in partnership with Takeda Canada, brought together healthcare professionals, policy makers, and patient advocates to discuss Canada's rare disease drug strategy, and how best to plan for the future ahead. In 2019, the Government of Canada committed to developing a National Strategy for Drugs for Rare Diseases. This welcomed commitment presents an opportunity to improve screening, diagnosis, and treatments for Canada's rare disease population.

Opening Remarks: Building a National Strategy for Drugs for Rare Diseases

Michelle Boudreau,
Executive Director, Office of Pharmaceutical Management Strategies
Health Canada

- Michelle Boudreau gave an overview of the current state of the strategy in government.
- There is a strong appetite for collaboration by the provinces and territories on this important initiative.

"Patients, their families, and their caregivers voiced that investments in governance, infrastructure and evidence generation are critical for a successful national strategy."

Panel I: The Rare Disease Environment in Canada

- The discussion was moderated by **Marcel Saulnier,** former Associate Assistant Deputy Minister (ADM) for Health Canada and current Associate with Santis Health.
- Canada has made significant progress in recent years in increasing access to treatments and care for rare disease patients.
- **Findings:** Canada must address shortcomings where efforts must be focused.
- Health Canada faces the challenge of incorporating the diverse needs and viewpoints of the array stakeholders in the rare disease space in a holistic strategy.
- The panel remained optimistic and united in the common goal of increasing access to care and treatments for Canadians living with rare diseases

Michelle Mujoomdar,
Director, Specialty Pharmaceuticals,
Health Canada

Suzanne McGurn,
President and CEO,
CADTH

Durhane Wong-Rieger,
President & CEO,
Canadian Organization for Rare Disorders

Dr. Aneal Khan,
Medical Geneticist

“Everyone has great ideas and it’s about really trying to coalesce around a common vision and a plan for a way forward.” - Michelle Mujoomdar

“We really need to make sure that we've got a health system and we've got health services that are lined up with being able to optimize bringing in drugs." - Durhane Wong-Rieger

“We are very alert to how we balance best practises (from other jurisdictions) with learnings from things that didn’t work so well.”- Suzanne McGurn

“We need regulations and accountability for the regulations, so that a person with a rare disease doesn’t have to worry about getting access to the drug. At the same time we need a path for us to say that the drug has to be used in a way that works.”- Dr. Aneal Khan

Panel II: Preparing for the Future

- The discussion was moderated by **Francis Drouin,** MP for Glengarry—Prescott—Russell (Ontario), rare disease advocate and chair of the Federal ALS Parliamentary Caucus.
- **Findings**: Canada should incorporate key success factors from comparable jurisdictions, adapting them to the Canadian context to ensure an effective strategy.
- Critical improvements are needed in early diagnosis and screening; research; standards of care; clinical criteria for treatment eligibility and early and equitable access; data infrastructure for real-world evidence; and centers of excellence.

Rute Fernandes, General Manager, Takeda Canada

Dr. Stephen Betschel, Chair of the Canadian Hereditary Angioedema Network, medical advisor for Immunodeficiency Canada, and HAE Canada

Whitney Goulstone, Executive Director, Canadian Immunodeficiencies Patient Organization

Jo De Cock, Previous CEO of INAMI-RIZIV, RWE4decisions

“If all stakeholders together prioritize rare diseases as key to improve health care system in Canada, Canada can leapfrog and can really be a reference globally, from diagnosis to treatment and to research, it is a shared responsibility, and we can make a meaningful impact." -Rute Fernandes

"Having the engagement and discussion and bringing people together early on to try to understand how to set this up." -Dr. Stephen Betschel

“Only interactive dialogue can help us make progress in confronting this gap for patients with rare diseases.” -Jo De Cock

“The patient journey needs to be remembered. There are huge gaps before we get to the treatment…we are looking at newborn screening, we are looking at diagnosis where we are looking at gaps of up to 12 years in certain rare disease and in some provinces, there aren’t even treatment centers. - Whitney Goulstone